Development of the Theracyte Cellular Encapsulation System for Delivery of human ES Cell-derived Pancreatic Islets and Progenitors.

Grant Award Details

Development of the Theracyte Cellular Encapsulation System for Delivery of human ES Cell-derived Pancreatic Islets and Progenitors.

Grant Type: Tools and Technologies I

Grant Number: RT1-01093

Investigator:

Name:	Evert Kroon
Institution:	ViaCyte, Inc.
Type:	PI

Disease Focus: Diabetes, Metabolic Disorders

Human Stem Cell Use: Adult Stem Cell, Embryonic Stem Cell

Award Value: $827,072

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 2

View Report

Grant Application Details

Application Title: Development of the Theracyte Cellular Encapsulation System for Delivery of human ES Cell-derived Pancreatic Islets and Progenitors.

Public Abstract:

There are several challenges to the successful implementation of a cellular therapy for insulin dependent diabetes derived from Human Embryonic Stem Cells (hESCs). Among these are the development of functional insulin-producing cells, a clinical delivery method that eliminates the need for chronic immunosuppression, and assurance that hESC-derived tumors do not develop in the patient.

We have recently developed methods to efficiently generate such insulin-producing cells from Human Embryonic Stem Cells that can prevent diabetes in mouse models of the disease. The results demonstrated for the first time that Human Embryonic Stem Cells could indeed serve as a source of cellular therapy for diabetes. However, the clinical use of Human Embryonic Stem Cell-derived cell products is hampered by safety concerns over the potential growth of unwanted cell types and the formation of tumors.

Encapsulation of cellular transplants has the potential to reduce or eliminate the need for immunosuppression. Moreover, a durable immunoprotective device which prevented cell escape could serve as a platform for safely administering Human Embryonic Stem Cell-derived therapies. The [REDACTED] device, a planar polytetrafluorethylene (PTFE) pouch-like encapsulation device, features 100% encapsulation and is fully retrievable. We and others have demonstrated in various animal models that the device provides obust protection of transplanted cells against immune attack from the host, [REDACTED] -encapsulated insulin-producing cells can correct diabetes in animals, and the device can prevent the escape and spread of cancer cells.

Therefore, the goal of the proposed studies is to evaluate the retrievable [REDACTED] cell encapsulation device in combination with Human Embryonic Stem Cell-derived pancreatic progenitor cells for the treatment of diabetes in mice.

Statement of Benefit to California:

With a current prevalence of greater than 170 million individuals world-wide, diabetes has attained epidemic proportions. The widespread secondary complications of kidney failure, cardiovascular disease, peripheral nerve disease, and severe retinopathies, this disease extracts a relentless and costly toll on the patients and the health care establishments required for their treatment. Current estimates are that California spends minimally $12 billion on diabetes not including lost wages. There are more than 300,000 diabetes related hospitalizations costing $3.4 billion annually. To date, cellular replacement has been performed either by transplantation of whole pancreas organs, or via infusion of isolated primary pancreatic islets into the portal vein . While effective, the availability of such procedures is severely limited for the treatment of the general diabetes population since it relies upon the extremely limited supply of pancreas organs from deceased donors and usually requires life-long administration of immuno-suppressive drugs.

Recent advances in human embryonic stem cell research indicate that the production of a virtually unlimited supply of functional insulin-producing cells is possible. However, much research is required to determine how to safely administer such cells as a therapy because they could give rise to tumors. The animal studies proposed in this application address this with a device that could both protect the therapeutic cells from host immune attack, and protect the host from tumor formation. If successful, our research would provide a possible opportunity for safely administering a diabetes therapy derived from human embryonic stem cells.
